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# ICER 2020-2023 Value Assessment Framework

**Overview of Conceptual Elements and Procedures**

**January 31, 2020**



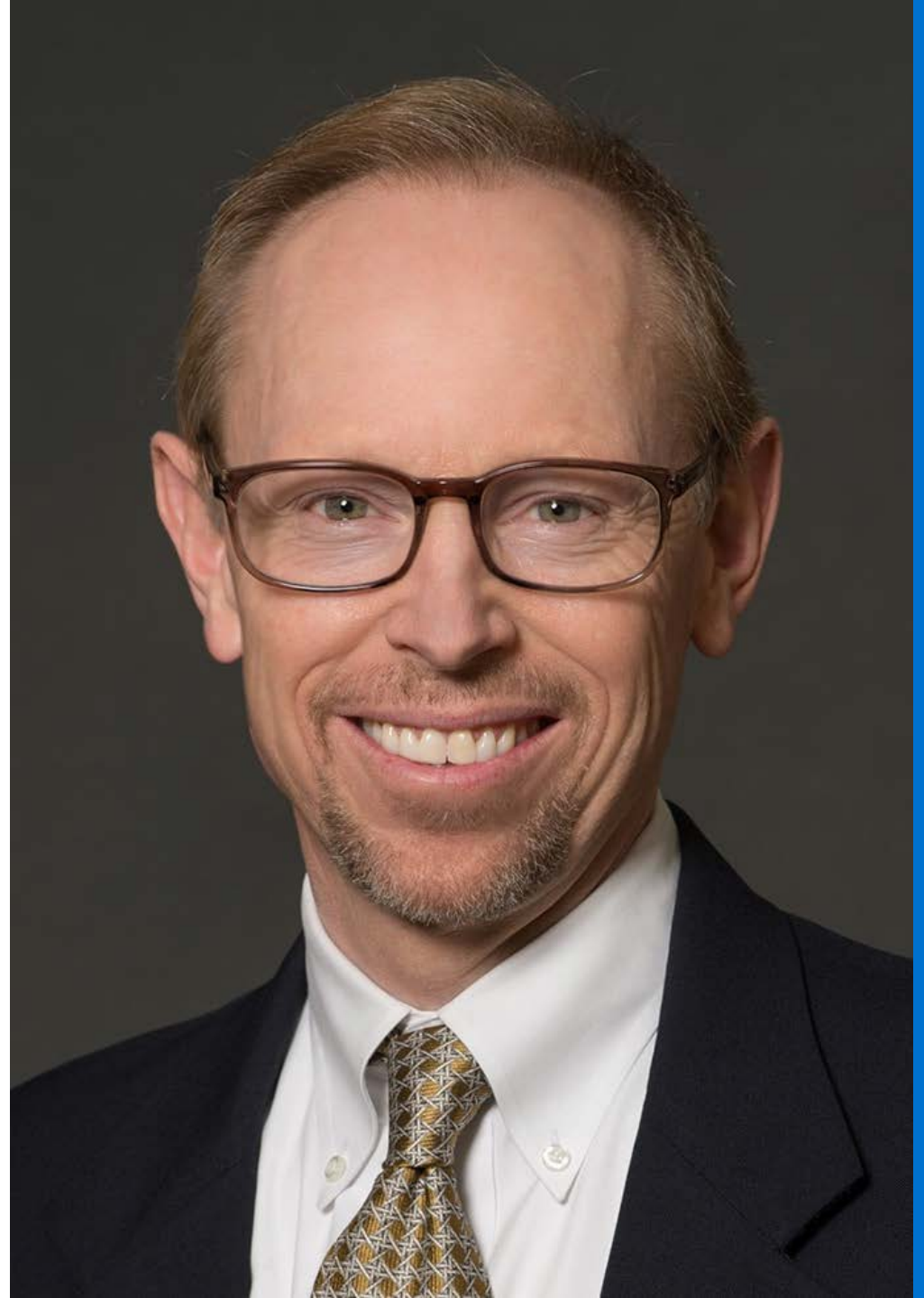
**Steven D. Pearson MD, MSC**

President



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## Webinar Goals

- ICER's guiding principles for HTA
- Conceptual elements of ICER Value Assessment Framework
- Procedures for Report Generation, Stakeholder Engagement, and Public Meetings
- More detailed descriptions can be found in the [ICER Value Assessment Framework](#) and other [methods documents](#) and [engagement guides](#)

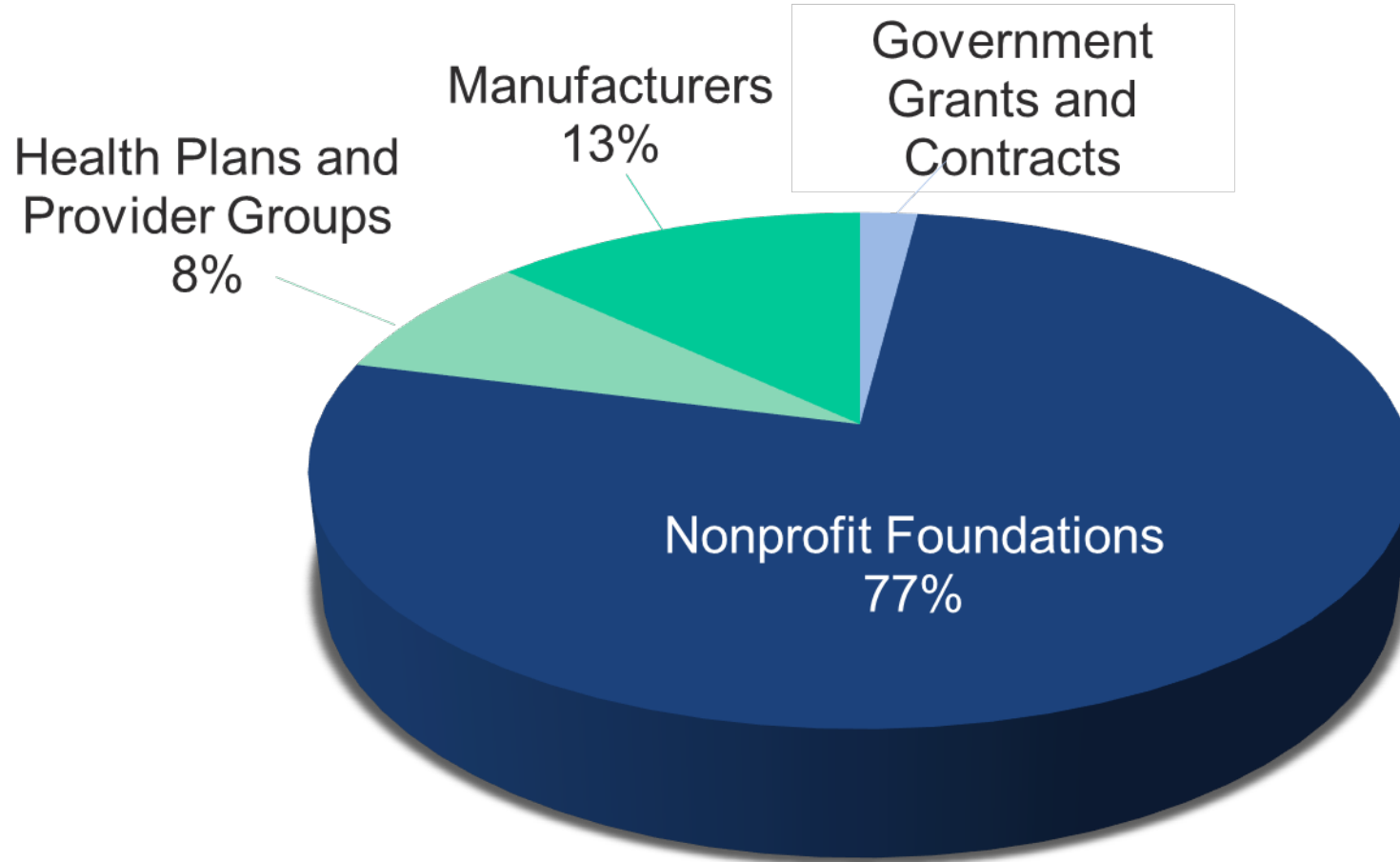
# About ICER

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# Institute for Clinical and Economic Review (ICER)

- **Independent** health technology assessment group
- Develop **publicly available value assessment** reports on medical tests, treatments, and delivery system innovations
- Convene regional **independent appraisal committees** for public hearings on each report

# 2019 Funding Sources



■ ICER Policy Summit and Non-Report activities only

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# Guiding Goals and Principles:

## Fair Prices, Fair Access, Future Innovation, Fair Deliberation

- Fair Price
  - Aligning price with added benefit for patients is the best way to assure health care resources achieve the maximum health without doing more harm than good
- Fair Access
  - Applying evidence and considerations of value to coverage should not be done primarily to shift costs onto patients or to use burdens of coverage authorization to limit appropriate access
- Future Innovation
  - Both patients and the public want a thriving ecosystem for innovation that brings demonstrable benefits to patients – at prices that patients and the system can afford
- Fair Deliberation
  - Respect for all
  - Clear and Inclusive Procedures
  - Transparent Decisions

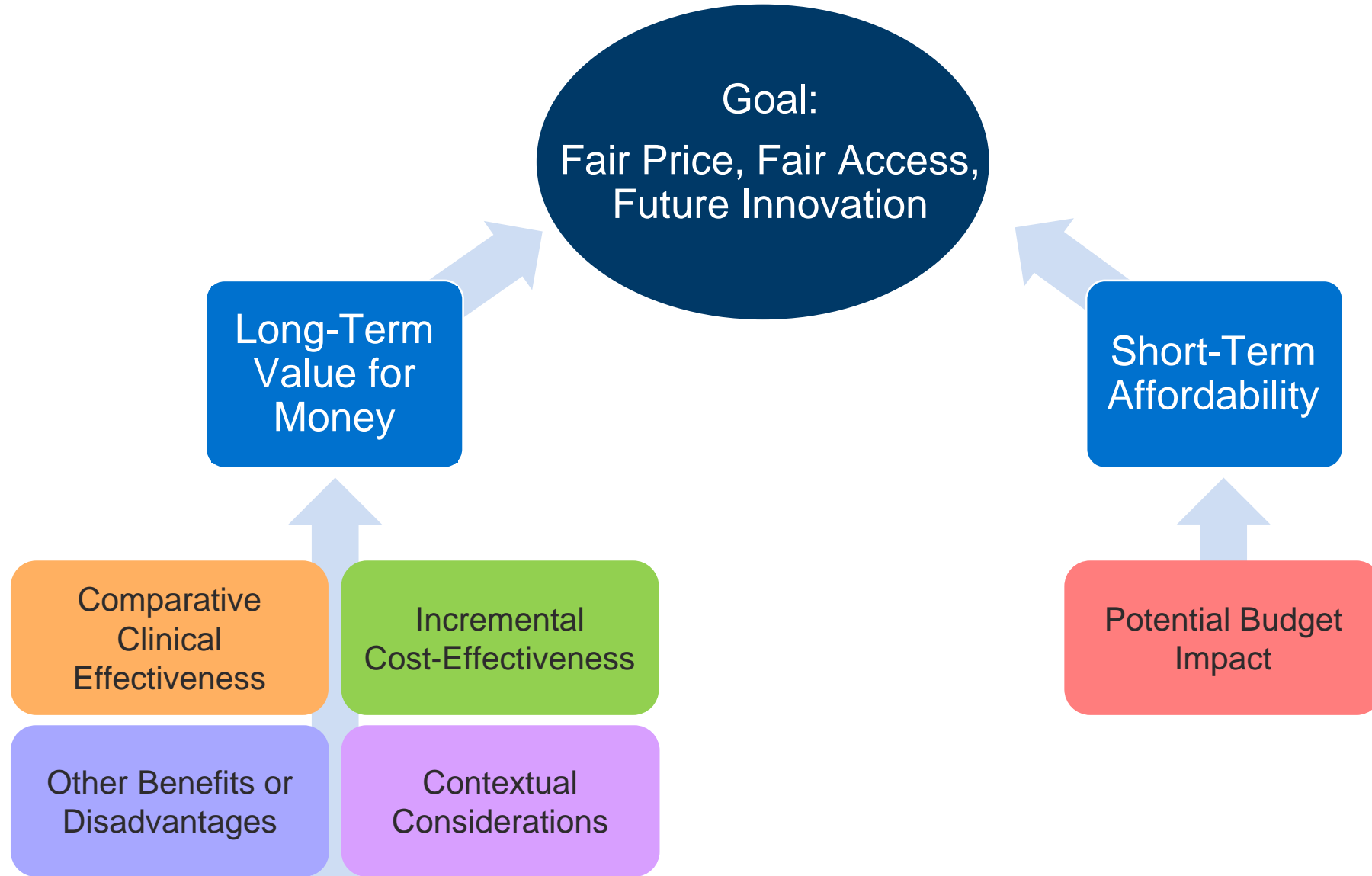
# Conceptual Elements of Value Assessment Framework



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# The ICER Value Framework: Purpose

- Intended to make transparent how “value” is conceived of and evaluated in ICER reports
- Takes a “population” level perspective as opposed to trying to serve as a shared decision-making tool to be used by individual patients and their clinicians
- Even with its population-level focus, the ICER value framework seeks to encompass and reflect the experiences and values of patients



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# Key Features of ICER Value Assessment Framework

- Anchoring in long-term perspective on value
- Foundation in evidence on comparative clinical effectiveness
- Acceptance of multiple forms of evidence
- Recognition that what matters to patients is not limited to measured “clinical” outcomes
- Acknowledgment of the role of contextual considerations in value judgments
- Inclusion of information on potential budget impact

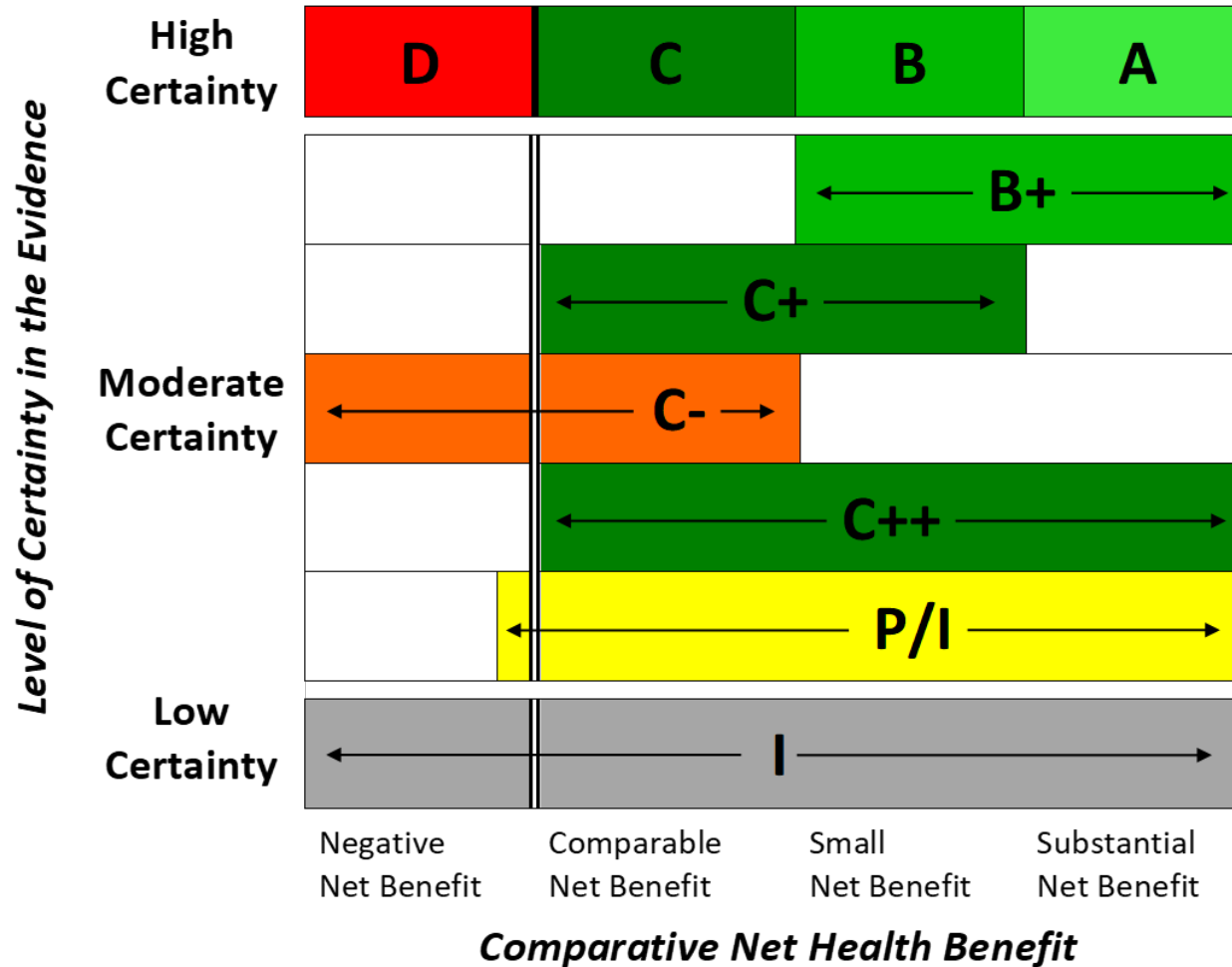
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# Comparative Clinical Effectiveness

- Systematic review of evidence
  - Patient groups inform what outcomes are important to them; differences across severity, time in disease course, etc.
  - Publicly-available material, possible generation of new data from patients and caregivers, and option for in-confidence material from manufacturers
  - Evidence sought from multiple sources, not just randomized controlled trials (RCTs)
  - Direct comparisons or indirect comparisons through network meta-analysis
  - Examination of subgroups and heterogeneity of treatment effect
  - ICER EBM Rating Matrix to present ratings of comparative clinical effectiveness

# ICER EBM Rating Matrix

Comparative Clinical Effectiveness



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# Incremental Cost-Effectiveness

- Computer modeling comparing long-term clinical outcomes and costs for different treatment options
- Costs from health system perspective – all health care costs
- Standard measures of health outcomes
  - Functional improvement, prevented negative outcomes
  - Additional life-years gained
  - Improvement in quality of life
  - Summing these for comparison across treatments: quality-adjusted life years (QALYs) and equal value of life years gained (evLYGs)

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## The core measure of health gain for patients: The Quality Adjusted Life Year (QALY)

- The **QALY** is recommended by US and international bodies
- A measure used to compare two or more treatments by comparing treatments impact on length and quality of life
  - Evidence on differential impact on length and quality of life comes from clinical studies
  - Clinical and functional outcomes (e.g. pain scores, ability to walk) have to be translated into quality of life when quality of life not measured directly
- Whose opinion is used to say how good it is to get better (e.g. regain sight) or to get worse (e.g. lose the ability to hear)?

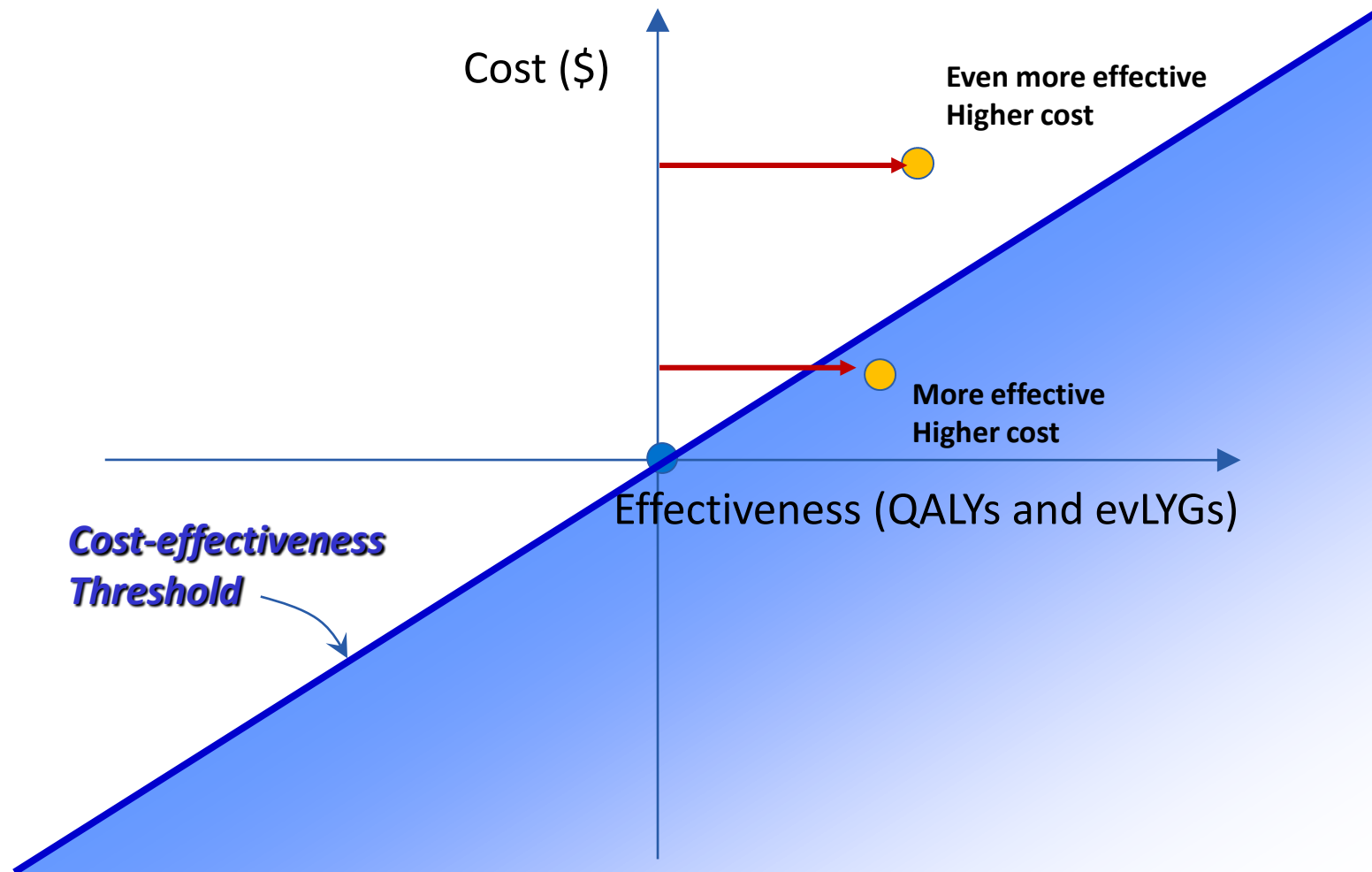
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# Equal Value of a Life Year Gained (evLYG)

- Complementary to the QALY
- Still captures differential effects of treatments on length of life and quality of life
- BUT -- if the treatment extends life, all extended time weighted as if full quality of health, i.e. “one day of extra life is valued the same no matter at what quality of life”
- Eliminates any risk that treatments that extend life at lower function will receive lower value-based price benchmarks
- Downside is that evLYG can override patients’ own valuation that time spent at much higher function is worth more than extended time at lower function



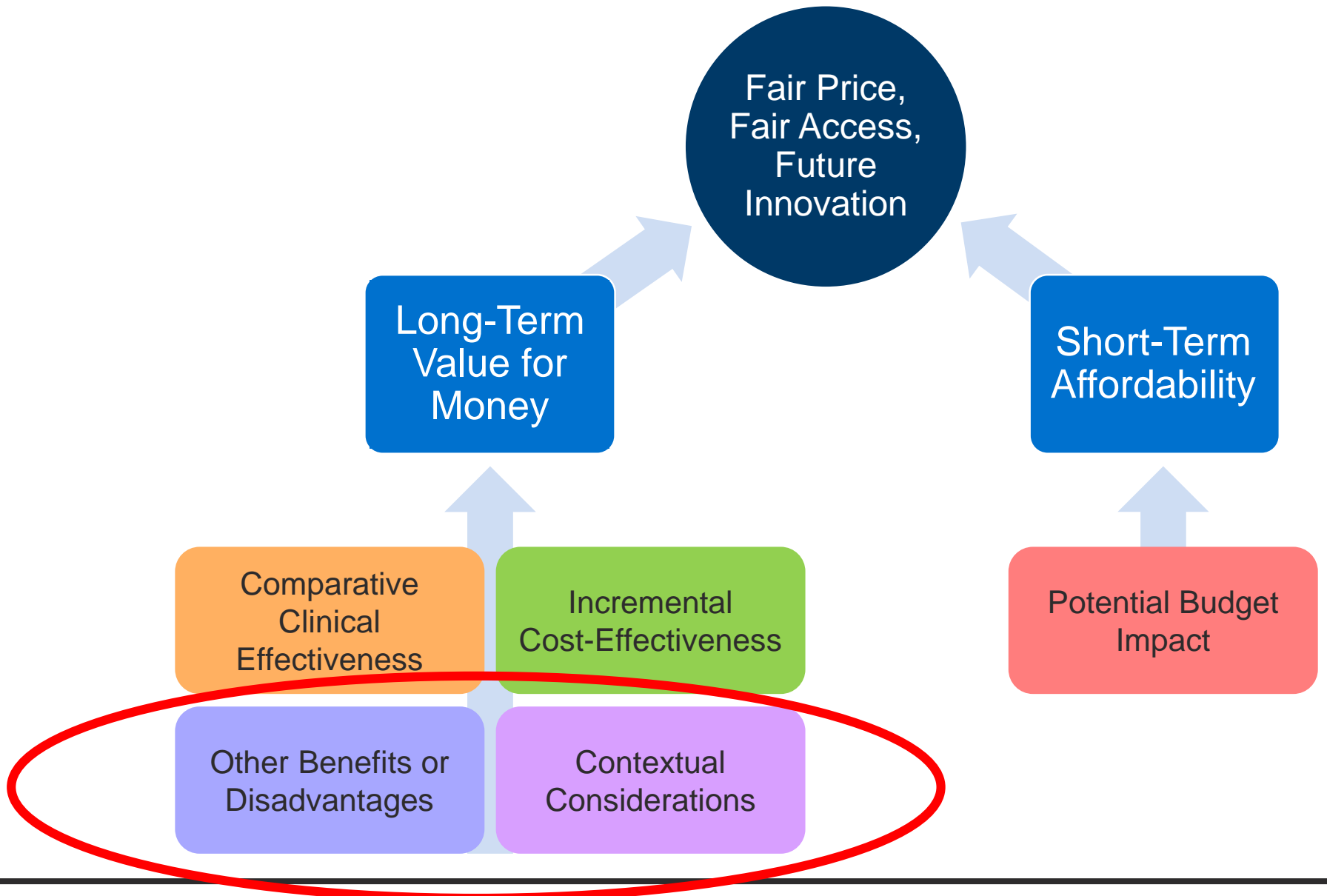
# Determining health benefit price benchmarks



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# Cost per QALY Thresholds

- Societal “willingness to pay”
  - WHO and ACC 1-3x per capita GDP; research ~\$100,000 per QALY
- Opportunity cost for the health system
  - ~\$15,000 per QALY in UK; \$84,000 per QALY in the US
- ICER reports: \$50,000-\$200,000 per QALY
  - Health benefit price benchmark: \$100-\$150K/QALY and per evLYG



## Potential Other Benefits or Disadvantages and Contextual Considerations

1 (Suggests Lower Value)	2 (Intermediate)	3 (Suggests Higher Value)
Uncertainty or overly favorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too optimistic		Uncertainty or overly unfavorable model assumptions creates significant risk that base-case cost-effectiveness estimates are too pessimistic
Very similar mechanism of action to that of other active treatments		New mechanism of action compared to that of other active treatments
Delivery mechanism or relative complexity of regimen likely to lead to much lower real-world adherence and worse outcomes relative to an active comparator than estimated from clinical trials		Delivery mechanism or relative simplicity of regimen likely to result in much higher real-world adherence and better outcomes relative to an active comparator than estimated from clinical trials
Will not have a significant impact on improving return to work and/or overall productivity versus the comparator		Will have a significant impact on improving return to work and/or overall productivity versus the comparator
This intervention will not differentially benefit a historically disadvantaged or underserved community		This intervention will differentially benefit a historically disadvantaged or underserved community
Small health loss without this treatment as measured by absolute QALY shortfall		Substantial health loss without this treatment as measured by absolute QALY shortfall
Small health loss without this treatment as measured by proportional QALY shortfall		Substantial health loss without this treatment as measured by proportional QALY shortfall
Will not significantly reduce the negative impact of the condition on family and caregivers versus the comparator		Will significantly reduce the negative impact of the condition on family and caregivers versus the comparator
The intervention offers no special advantages to patients by virtue of presenting an option with a notably different balance or timing of risks and benefits		The intervention offers special advantages to patients by virtue of presenting an option with a notably different balance or timing of risks and benefits
Other		Other

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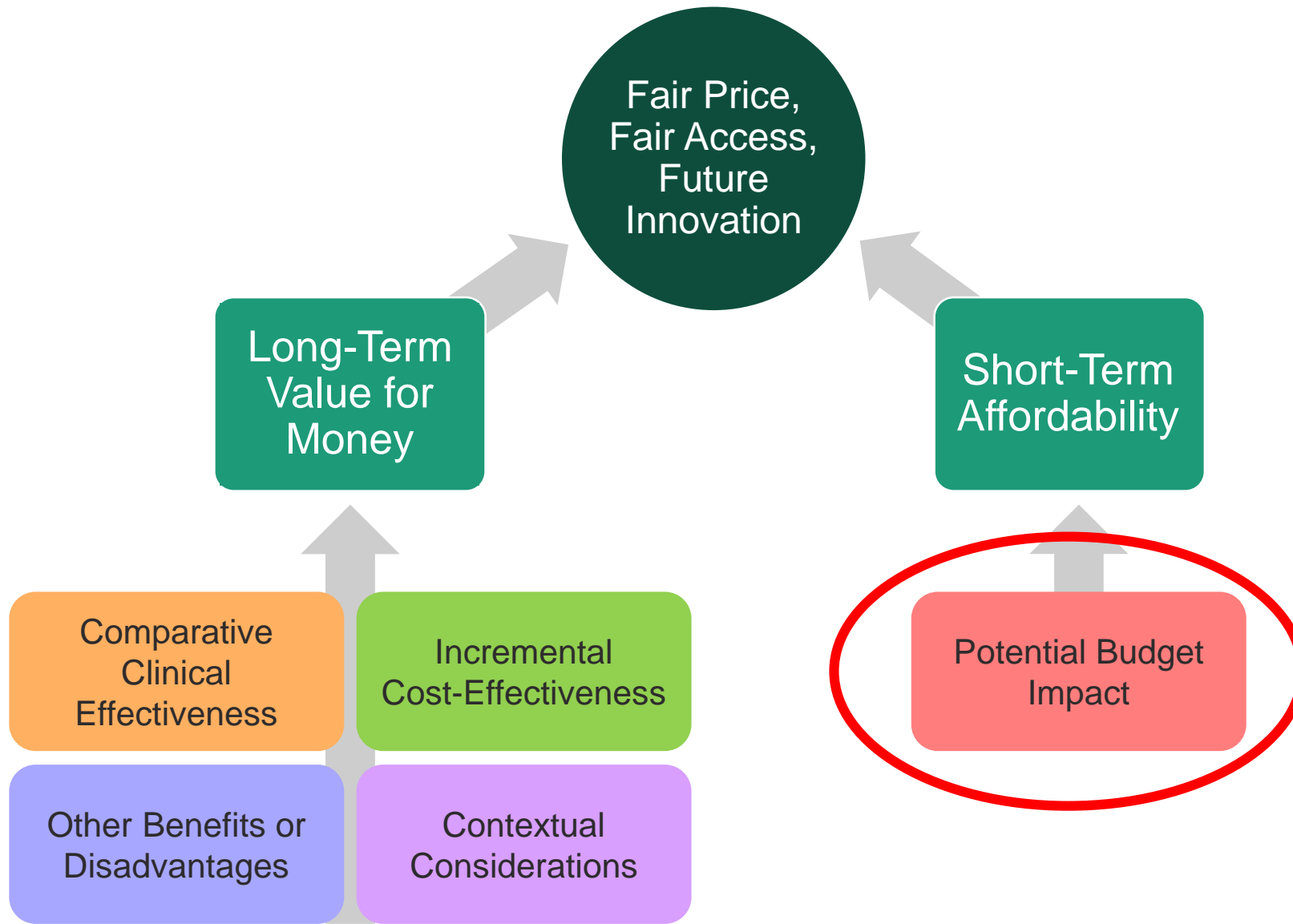
# What is the intended role of votes on POBs/CCs?

**1. Consider Health Benefit  
Price Benchmark Range**

**2. Apply Potential Other Benefits  
or Disadvantages and  
Contextual Considerations**

Price to reach  
\$100k/QALY or evLYG

Price to reach  
\$150k/QALY or evLYG



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# Potential Budget Impact Threshold

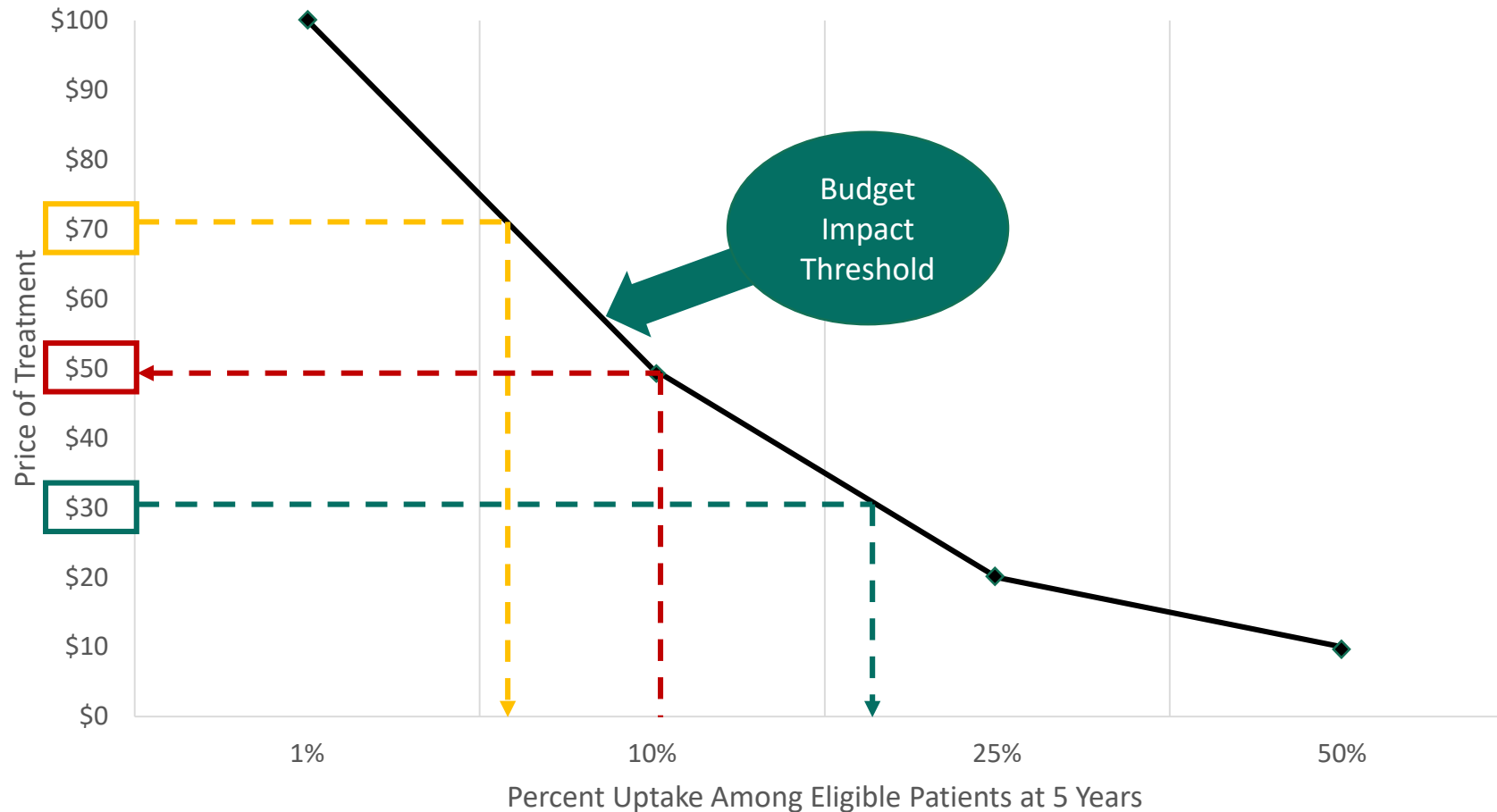
- Signal to stakeholders and policy makers when the amount of added health care costs associated with a new service – even one with good long-term value -- may be difficult for the health system to absorb over the short term without displacing other needed services or contributing to rapid growth in health care insurance costs that threaten access and affordability.
- Example: Sovaldi for Hepatitis C
- **NOT A BUDGET CAP!**



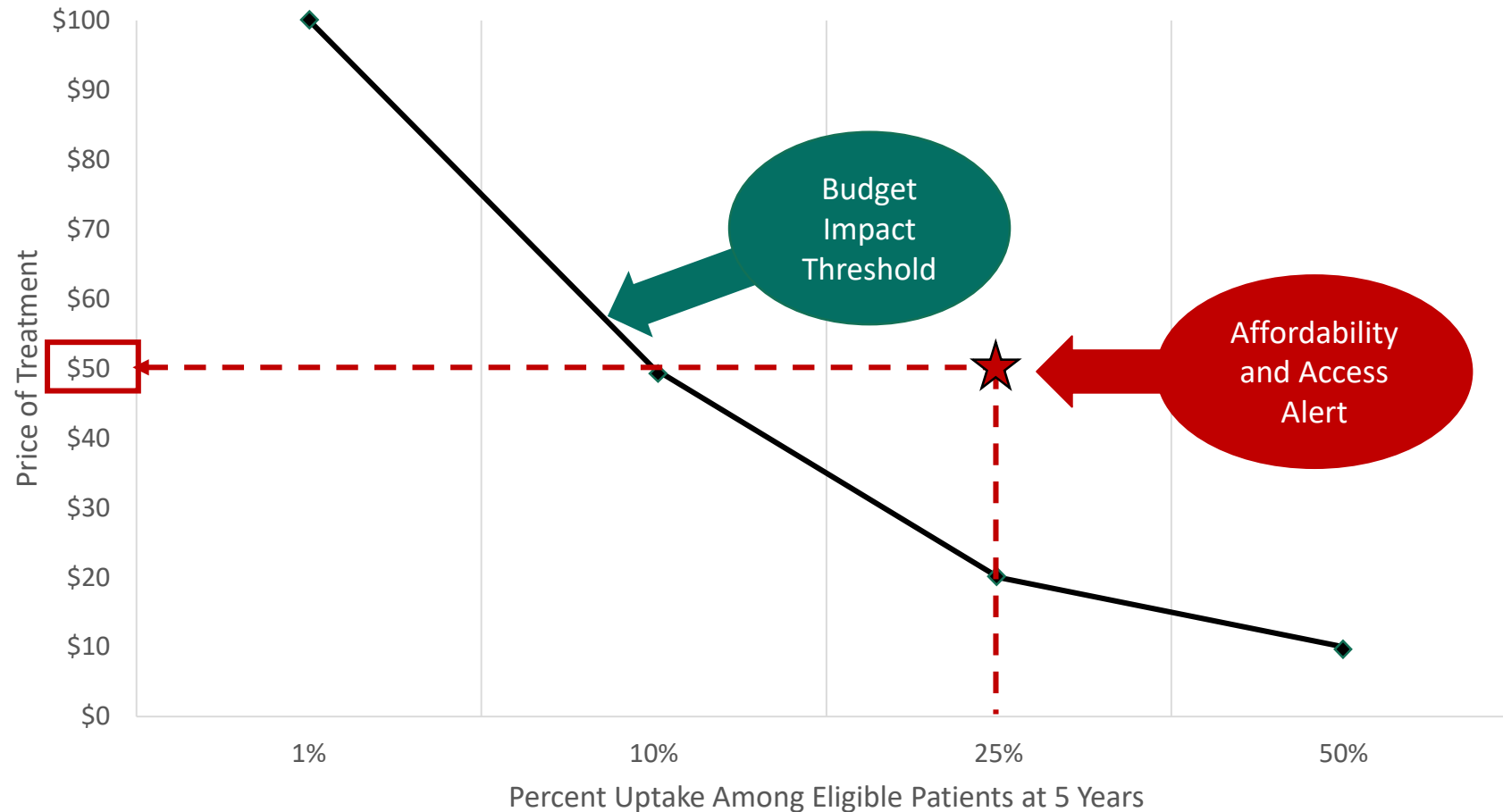
# Potential Budget Impact Threshold, 2019

Item	Parameter	Estimate	Source
1	Growth in US GDP + 1%	3.5%	World Bank, 2019
2	Total personal medical care spending, 2018 estimate	\$2.95 trillion	CMS National Health Expenditures, 2019
3	Contribution of drug spending to total health care spending (%) (Row 4 ÷ Row 2)	16.9%	Calculation
4	Contribution of drug spending to total health care spending, 2018	\$498.6 billion	CMS National Health Expenditures, 2019; Altarum Institute, 2018
5	Annual threshold for net health care cost growth for ALL drugs (Row 1 x Row 4)	\$ 17.4 billion	Calculation
6	Average annual number of new molecular entity approvals over 5 years (2014-2018)	42.6	FDA, 2019
7	Annual threshold for average cost growth per individual new molecular entity (Row 5 ÷ Row 6)	\$409.6 million	Calculation
8	Annual threshold for estimated potential budget impact for each individual new molecular entity (Doubling of Row 7)	\$819 million	Calculation

# Potential Budget Impact Scenarios



# Potential Budget Impact Scenarios



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# Key Modifications for Treatments of Ultra-Rare Disorders

- Eligible treatments
  - Condition/label for less than approximately 10,000 individuals
  - No ongoing or planned clinical trials for patient populations greater than 10,000
- ICER will provide specific context regarding the potential challenges of generating evidence
  - Conducting RCTs
  - Validating surrogate outcome measures
  - Obtaining long-term data on safety and on the durability of clinical benefit.
  - Commonly-used approach of evaluating treatments for URDs against historical controls will be highlighted

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## Key Modifications for Treatments of URDs

- Value-based price benchmark remains \$100-\$150K/QALY
  - Specific language included regarding decision-makers' history of accepting higher thresholds
- Manufacturers invited to supply information on costs of research and development if they desire

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# Modifications for High-Impact Single or Short-Term Therapies (SSTs)

- Why any modification?
  - Highly valued – in principle -- by society
  - Increased uncertainty with unrecoverable costs
  - Specific challenges to traditional cost-effectiveness modeling
- Eligible Treatments
  - One-time treatments that are potentially curative
  - Short-term treatments that offer transformative benefits (i.e., halt disease progression)

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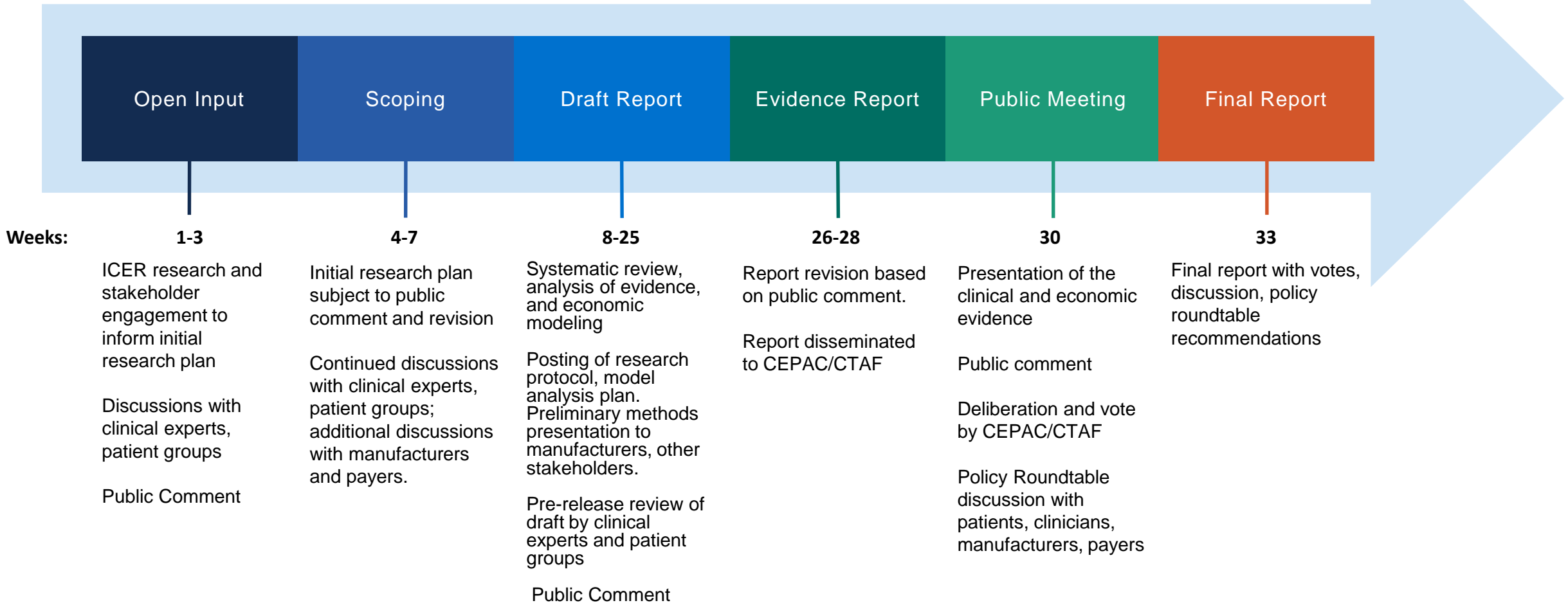
# Key Modifications for SSTs

- Additional modeling scenarios
  - Optimistic/conservative benefit (duration, magnitude, quality)
  - Threshold analysis for durability of effect
  - Hypothetical shared savings analyses
    - 50% of cost offsets assigned to health system
    - Cost offsets capped at \$150k but otherwise are assigned fully to treatment)

# Processes and Public Procedures



# ICER Report Development Process: 8 Months\*



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# Patient Engagement Program

- Annual calls/meetings with patient groups for major therapeutic classes (RA, psoriasis, MS, etc.) and early outreach to other patient groups when ICER has high certainty of review. Goal is to provide early guidance on ICER process and help facilitate empowered engagement.
- At time of topic announcement work with patient groups to identify most important outcomes for patients and determine if/how patient groups can contribute empirically to the economic model
- For key outcomes without data work with patient groups to identify potential RWE sources and/or develop patient survey
- At conclusion of the review invite patient groups for formal debrief on the experience
- At conclusion of the review offer to co-write and promote a letter to FDA and other stakeholders with proposals for improving the generation of patient-relevant data as part of the drug development process

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# Independent Appraisal Committees Meet 3-4x per year



 **CTAF**  
CALIFORNIA TECHNOLOGY  
ASSESSMENT FORUM

 **MIDWEST**  
**CEPAC**  
COMPARATIVE EFFECTIVENESS  
PUBLIC ADVISORY COUNCIL

 **NEW ENGLAND**  
**CEPAC**  
COMPARATIVE EFFECTIVENESS  
PUBLIC ADVISORY COUNCIL

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# Appraisal Committee Meeting Process

- CEPAC receives Evidence Report following public comment period on Draft Evidence Report
- Call with clinical experts one week before meeting
- Public meeting
  - Evidence synopsis on effectiveness and cost- effectiveness presented by lead reviewer
  - Patient representatives and clinical experts available to comment/respond to questions
  - Manufacturer comment
  - Patient and public comment
  - Discussion and voting on comparative clinical effectiveness, “other benefits and contextual considerations”, and long-term value for money at current prices.
  - Policy Roundtable discussion among key stakeholders

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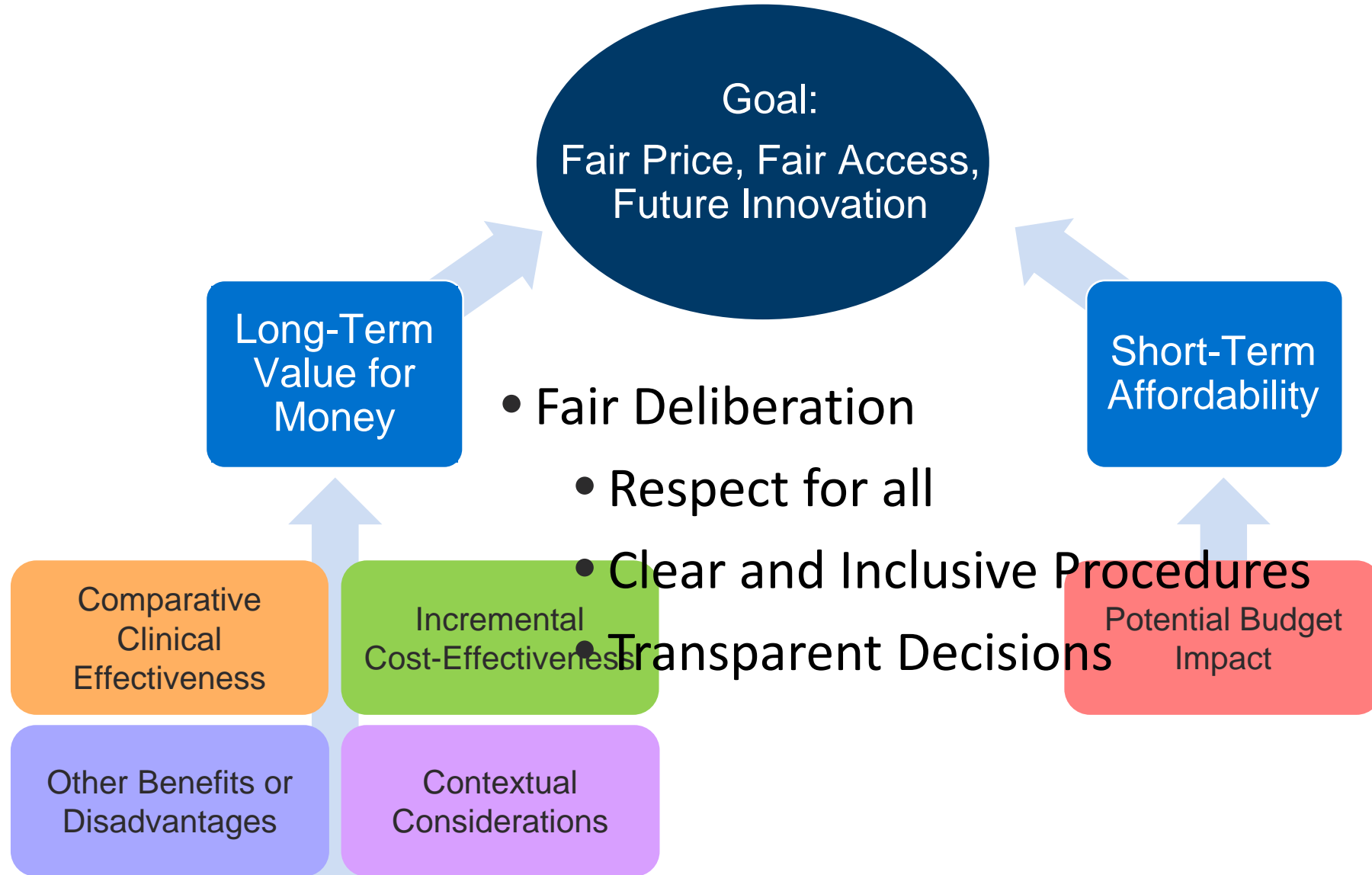
# ICER Final Report

- Final report and associated summaries include
  - Results of appraisal committee voting with commentary
  - Synopsis of key themes of policy roundtable discussion
  - Policy perspectives and recommendations related to application of evidence to clinical care, insurance coverage, pricing, and future research needs

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# Report Updates

- 12-Month Report Check-Ups
  - Outreach to stakeholders at 12-month report anniversary to identify new data that may impact report conclusions, update systematic review if evidence identified
  - Mark initial review as still / no longer current
  - Review enters queue for formal update alongside other potential assessments
- 24-Month RWE Update Pilot
  - At 2-year anniversary, collaborate with external partner to analyze RWE for select drugs approved under accelerated pathways



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# Thank you

